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## Neuroprotection to treat Alzheimer's: a new paradigm using human central nervous system cells

### Grant Award Details

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Neuroprotection to treat Alzheimer's: a new paradigm using human central nervous system cells

**Grant Type:** Disease Team Therapy Planning I

**Grant Number:** DR2-05416

**Investigator:**

**Name:** Alexandra Capela

**Institution:** StemCells, Inc.

**Type:** PI

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**Disease Focus:** Alzheimer's Disease, Neurological Disorders

**Award Value:** \$90,101

**Status:** Closed

### Progress Reports

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**Reporting Period:** Year 1

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### Grant Application Details

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**Application Title:** Neuroprotection to treat Alzheimer's: a new paradigm using human central nervous system cells

**Public Abstract:**

Alzheimer's disease (AD) is an incurable disorder that affects memory, social interaction and the ability to perform everyday activities. In the USA alone, the number of AD patients aged 65 and older has surpassed 5 million and that number may triple by 2050. Annual health care costs have been estimated to exceed 172 billion dollars, but do not reflect loss of income and stress caused to caregivers. Therefore, there is great hope for new therapies that will both improve symptoms and alleviate suffering.

There are few FDA-approved medications to treat AD and none is capable of preventing, delaying onset or curing AD. Current medications mostly tend to temporarily slow the worsening of AD-associated symptoms such as sleep disturbances, depression and memory loss/disorientation. Pharmaceutical companies continue to develop new types of drugs or combination therapies that can better treat the symptoms or improve the quality of life of AD patients. There is also an ongoing effort to discover novel drugs that may prevent, reverse, or even cure AD. Unfortunately, the number of clinical studies addressing the possible benefit of such drugs is low, and agents that have shown initial promise have failed at later stage clinical testing, despite convincing preclinical data. There are ongoing studies in AD patients using vaccines and other biological compounds but it is unclear when data from these new trials will be available and more importantly, whether they will be successful. The need for divergent and innovative approaches to AD is clearly suggested by the failure of experimental drugs.

Our proposal is to use brain stem cells to treat AD. This is a completely different approach to the more standard therapies described above such as drugs, vaccines, etc., and one that we hope will be beneficial for AD patients as a one-time intervention. AD is characterized by a dysfunction and eventual loss of neurons, the specialized cells that convey information in the brain. Death or dysfunction of neurons results in the characteristic memory loss, confusion and inability to solve new problems that AD patients experience. It is our hope that stem cells transplanted into the patient's brain may provide factors that will protect neurons and preserve their function. Even a small improvement in memory and cognitive function could significantly alter quality of life in a patient with AD.

**Statement of Benefit to California:**

Of the 5.4 million Americans affected with AD, 440,000 are California residents and, according to the Alzheimer's Association, this number is projected to increase between 49.1 - 81.0% (second highest only to Northwestern states) between 2000 and 2025. Given that California is the most populous state, AD's impact on state finances is proportionally high and will only increase as the population ages and AD incidence increases. The dementia resulting from this devastating disease disconnects patients from their community and loved ones by eroding memory and cognitive function. Patients gradually lose their ability to drive, work, cook and even carry out simple everyday tasks, and become totally dependent on others. The quality of life of AD patients is hugely affected and the burden on their families and caregivers is very costly to the state of California.

There is no cure for AD and no way to prevent it. Most approved therapies only address symptomatic aspects of AD and disease modifying drugs are currently not available. By enacting Proposition 71, California voters acknowledged and supported the need to investigate the use of novel stem cell based therapies to treat currently incurable diseases such as AD. Our goal is to leverage our proven expertise in developing neural stem cell based therapies for human neurodegenerative disorders and apply it to AD. We propose that neural stem cell transplantation into select regions of the brain will have a beneficial impact on the patient. If successful, a single intervention may be sufficient to delay or stop progression of neuronal degeneration and preserve functional levels of cognition and memory. In a disease such as AD, any therapy that can exert even a modest impact on the patient's ability to carry out some daily activities will have an exponential positive effect not only on patients but also on families, caregivers and the health care system.

The potential economic impact of such type of therapeutic intervention for California could be tremendous, not only by reducing the high costs of care but also by becoming a vital world center for stem cell interventions in AD.

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